



Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in May 2009 on request of the Sponsor.

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Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of opebacan for the treatment of meningococcal disease

On 28 August 2006, orphan designation (EU/3/06/402) was granted by the European Commission to XOMA Ireland Ltd, Ireland, for opebacan for the treatment of meningococcal disease.

What is meningococcal disease?

Meningococcal disease describes infections caused by *Neisseria meningitides* bacteria. It is a serious bacterial infection. These bacteria may enter the cerebrospinal fluid (CSF) and irritate the meninges - the membranes that line the brain and spinal cord, causing inflammation of these membranes. This is known as meningitis. The most common symptoms of meningitis are headache and neck stiffness associated with fever, confusion, and an inability to tolerate bright light. Suspicion of meningitis is a medical emergency and immediate medical assessment is recommended.

Meningococcal disease can also lead to sepsis - a dangerous and potentially life-threatening blood infection. The infection is highly contagious; it can be spread by a cough or sneeze, or even a drink from a contaminated cup. The condition is chronically debilitating with severe complications, including hearing loss and brain damage. Meningococcal disease affects both children and adults and is life-threatening.

What is the estimated number of patients affected by the condition?

At the time of designation meningococcal disease affected less than 1 in 10,000 people in the European Union (EU)*. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of less than 46,000 people.

What treatments are available?

Several antibiotics were authorised for the condition in some countries of the Community. Moreover, a vaccine for meningococcal group C, considered responsible for majority of severe cases in the Community, is routinely administered in several EU Countries.

Opebacan has a different mechanism of action than the existing treatments and could thus be of potential significant benefit, particularly in cases of sepsis, when co-administered with antibiotics at the very early phase of the infection. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

* Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

How is this medicine expected to work?

Although antibiotics can kill bacteria, such as that caused by *Neisseria meningitides*, they do not alleviate inflammation that usually accompanies infections. Toxins shed from the invading bacteria trigger an inflammatory response. Cells of our immune system (body's natural defence mechanism against infection and disease) are recruited to the site of infection and can potentially harm the surrounding tissues. Endotoxin is a component of the cell wall of certain bacteria, known as gram-negative bacteria, which can be released in the body during an infection. Meningococcal sepsis is characterised by high levels of bacterial endotoxin in the blood. Opebacan is a protein proposed to bind to endotoxin. It is expected to clear the blood from endotoxin and to limit the damage.

What is the stage of development of this medicine?

The effects of opebacan have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with meningococcal sepsis were completed.

Opebacan was not authorised anywhere worldwide for meningococcal disease at the time of submission. Orphan designation of opebacan was granted in the United States for severe meningococcal disease.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 12 July 2006 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- and either the rarity of the condition (affecting not more than five in 10,000 people in the Community) or the insufficient returns on investment

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

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**Translations of the active ingredient and indication in all EU languages
and Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	Opebacan	Treatment of meningococcal disease
Czech	Opebacan	Léčba meningokokového onemocnění
Danish	Opebacan	Behandlingen af meningokoksygdom
Dutch	Opebacan	Behandeling van meningokokken-infectie
Estonian	Opebacan	Meningokokkhaiguste raviks
Finnish	Opebakaani	Meningokokkisairauden hoito
French	Opébacan	Traitement de la méningococcie
German	Opebacan	Behandlung von Meningokokken-Infektionen
Greek	Opebacan	θεραπεία της μηνιγγιτιδοκοκκικής νόσου
Hungarian	Opebacan	Meningococcus fertőzés okozta betegségek kezelése
Italian	Opebacan	Trattamento della meningite meningococcica
Latvian	Opebakāns	Meningokoku infekcijas ārstēšana
Lithuanian	Opebakanas	Meningokokinės infekcijos gydymas
Polish	Opebakan	Leczenie zapalenia opon mózgowo-rdzeniowych
Portuguese	Opebacan	Tratamento da doença meningocócica
Slovak	Opebakan	Liečba meningokokovej choroby
Slovenian	Opebakan	Zdravljenje meningokokne bolezni
Spanish	Opebacán	Tratamiento de la enfermedad meningocócica
Swedish	Opebacan	Behandling av meningokocksjukdom
Norwegian	Opebakan	Behandling av meningokokksykdom
Icelandic	Opebacan	Meðferð á meningókokka sjúkdómi